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Ultragenyx Announces Preliminary Data From Phase 1/2 Study of Recombinant Human Beta-Glucuronidase in Mucopolysaccharidosis 7

Data Presented at American College of Medical Genetics and Genomics (ACMG) Annual Clinical Genetics Meeting

NOVATO, Calif., March 27, 2014 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (Nasdaq:RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today announced the presentation of preliminary data from the Phase 1/2 study of recombinant human beta-glucuronidase (rhGUS, UX003), an investigational therapy for the treatment of mucopolysaccharidosis 7 (MPS 7, Sly syndrome).

The following abstract is being presented at the ACMG Annual Clinical Genetics Meeting in Nashville, Tennessee.

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Phase 1/2 Open-Label Study to Assess Safety, Efficacy, and Dose of Recombinant Human Beta-Glucuronidase (rhGUS) in Subjects with Mucopolysaccharidosis Type VII.

The Phase 1/2 open-label clinical study is assessing the safety, efficacy, and dose of rhGUS administered every other week via intravenous infusion in a 12-week primary analysis phase, which will be followed by dose-exploration and long-term extension.

Preliminary results from three patients who have been administered 2 mg/kg of rhGUS every other week for two, six, and 12 weeks show evidence of clearance of lysosomal storage as indicated by the decline in urinary glycosaminoglycan (GAG) excretion beginning at two weeks of treatment of approximately 30-50%.

At the 12 week assessment of the first patient, absolute liver size was reduced by approximately 11%. This represents a 46% decrease in the excess liver size above normal for age and gender. The remaining patients have not yet reached the 12 week time point for liver size assessment.

No serious adverse events were observed during up to 12 weeks of treatment, and no infusion-associated reactions were observed after a total of 13 infusions to date in these three subjects. The study will continue and additional 12-week interim data are expected in 2014. If the efficacy and safety results for all treated subjects are supportive and the dose confirmed, the company intends to initiate a pivotal Phase 3 study enrolling at least 12 patients in 2014.

About MPS 7

Mucopolysaccharidosis type 7 (MPS 7, Sly syndrome), originally described in 1973 by William Sly, M.D., is a rare genetic, metabolic disorder and is one of 11 different MPS disorders. MPS 7 is caused by the deficiency of beta-glucuronidase, an enzyme required for the breakdown of the glycosaminoglycans (GAGs) dermatan sulfate and heparan sulfate. These complex GAG carbohydrates are a critical component of many tissues. The inability to properly break down GAGs leads to a progressive accumulation in many tissues and results in a multi-system disease.

While its clinical manifestations are similar to MPS 1 and MPS 2, MPS 7 is one of the rarest among the MPS disorders. MPS 7 has a wide spectrum of clinical manifestations and can present as early as at birth. There are no approved therapies for MPS 7 today. The use of enzyme replacement therapy as a potential treatment is based on 20 years of research work in murine models of the disease. Enzyme replacement as a strategy is well established in the MPS field as there are currently four approved enzyme replacement therapies for other MPS disorders: MPS 1 (Aldurazyme®, laronidase), MPS 2 (Elaprase®, idursulfase), MPS 4A (Vimizim™, elosulfase alfa), and MPS 6 (Naglazyme®, galsulfase).

Ultragenyx initiated a Phase 1/2 study in the UK to evaluate the safety, tolerability, efficacy, and dose of intravenous administration of rhGUS in December 2013.

About Ultragenyx

Ultragenyx is a development-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with an initial focus on serious, debilitating metabolic genetic diseases. Founded in 2010, the company has rapidly built a diverse portfolio of product candidates with the potential to address diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no approved therapies.

The company is led by a management team experienced in the development and commercialization of rare disease therapeutics. Ultragenyx's strategy is predicated upon time and cost-efficient drug development, with the goal of delivering safe and effective therapies to patients with the utmost urgency.

For more information on Ultragenyx, please visit the company's website at www.ultragenyx.com.

Forward-Looking Statements

Except for the historical information contained herein, the matters set forth in this press release, including statements regarding Ultragenyx's plans, expectations, goals, objectives, milestones, clinical studies, and product development are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, future results, performance, or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainties inherent in the clinical drug development process, including the regulatory approval process, the timing of our regulatory filings, and other matters that could affect the availability or commercial potential of our drug candidate. Ultragenyx undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of the Company in general, see Ultragenyx's Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 24, 2014, and its future periodic reports to be filed with the Securities and Exchange Commission.

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